rAAV Compositions and Methods for Delivery of Human Factor VII Polypeptides and Treatment of Hemophilia A

Abstract

Disclosed are improved recombinant adeno-associated viral (rAAV) vector compositions useful in the delivery of antihemophilic factor polypeptides to selected mammalian host cells. The disclosed rAAV vector compositions preferably comprise one or more polynucleotide sequences that express one or more mammalian Factor VII proteins, polypeptides, peptides, a operably linked to one or more promoter and/or enhancer sequences that are capable of expressing the encoded antihemophilic therapeutics in cells suitably transformed with the disclosed rAAV vector constructs, virions, and viral particles comprising the contructs of interest. These compositions, and methods for their use, including the manufacture of medicaments, have desirable therapeutic and/or prophylactic efficacy in the amelioration, treatment, and/or prevention of a variety of diseases, disorders, and dysfunctions in selected mammals, and in particular, humans diagnosed with Factor VII deficiency and/or hemophilia A.